Phase 1 Study of Autologous CD4LVFOXP3 in Participants with IPEX Syndrome

Grant Award Details

Phase 1 Study of Autologous CD4LVFOXP3 in Participants with IPEX Syndrome

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-13259

Project Objective: To conduct first in human Phase 1, dose escalation, clinical trial to establish safety and feasibility of administering autologous FoxP3 engineered CD4 (Treg -like) cells in male patients with IPEX Syndrome.

Investigator:

Name:	Rosa Bacchetta
Institution:	Stanford University
Type:	PI

Disease Focus: Blood Disorders, IPEX Syndrome

Human Stem Cell Use: Adult Stem Cell

Award Value: $11,999,179

Status: Pre-Active

Grant Application Details

Application Title: Phase 1 Study of Autologous CD4LVFOXP3 in Participants with IPEX Syndrome

Public Abstract:

Therapeutic Candidate or Device

CD4+ T cells that have undergone lentiviral -mediated gene transfer of Forkhead Box P3 (FOXP3) and acquired regulatory T cell function.

Indication

Immune dysregulation Polyendocrinopathy Enteropathy X-linked (IPEX) syndrome

Therapeutic Mechanism

Administration of autologous CD4LVFOXP3 that constitutively and stably express wild-type FOXP3 gene will replace the lack of function regulatory T cells in patients with IPEX syndrome, a life-threatening pediatric disease due to FOXP3 gene mutation, and a prototype of genetic autoimmune disease.

Unmet Medical Need

IPEX has early severe onset and is a serious clinical challenge. Pharmacological immunosuppression can only partially control autoimmune manifestations and does not prevent organ damage. Allogeneic HSCT can cure but lack of suitable donors and transplant complications lead to inferior outcomes.

Project Objective

Phase 1 trial to select dose and safety

Major Proposed Activities

- Evaluate feasibility and safety (primary objectives)
- Explore the potential for clinical efficacy of CD4LVFOXP3 infusion on clinical disease manifestations (secondary objectives)
- Perform immune monitoring to establish immune criteria that predict successful patient outcomes.

Statement of Benefit to California:

IPEX syndrome impacts patients as well as families and communities. Thus, improved treatment for IPEX patients would have tremendous personal benefit and would provide a great economic benefit to the state by ensuring California is a beacon for this innovative treatment. A successful outcome of this Treg replacement therapy in IPEX could support development of CD4LVFOXP3 cell product for other diseases with autoimmunity and immune dysregulation (e.g. IBD, T1D, scleroderma, acute GVHD).
